Assessment in RCT's of Behavioral Interventions

Linking Hypotheses, Outcomes and Assessment Measures

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Principle I

- Study hypotheses should be framed so that they translate to measurable variables
  - Example:
    - Pts who receive Cognitive Adaptation Training will report fewer psychotic, depressive and negative symptoms, higher levels of adaptive functioning and lower rates of relapse

Principle II

- Variables should be operationally defined by assessment measures
  - Example:
    - psychotic, depressive and negative symptoms
      - Brief Psychiatric Rating Scale
    - adaptive functioning
      - Multnomah Community Adjustment Scale

Principle III

- Assessments should be defined in advance and linked to domains of outcome in the study protocol and operations manual
  - example
    - psychosis — BPRS items
    - — suspiciousness
    - — conceptual disorganization
    - — unusual thought content

Principle IV

- Timing of assessments should capture the time course of anticipated change
  - Example - CARS clinical trial
    - psychosis symptoms measured weekly for first five weeks, then monthly
    - cognitive functions measured at baseline and six months

Principle V

- Assessment instruments should be "attractive" to reviewers and assessors
  - Established , with a history of use in the field
  - Document reliability
    - Published inter-rater
    - Study inter-rater
  - Document validity
    - Face
    - Discriminant

Universal Assessment Measures

- Definition of patient population
- Information regarding “Refusers”
- Randomization
- “Housekeeping”
- Adverse Events/Intercurrent Medical Illness
- Quality of Life

Definition of Patient Population

- Inclusion/exclusion criteria
- Diagnosis
- Other defining characteristics
- Refusers and generalization

Characterizing the Trial Population

- Outcome modifieres
  - Demographic information - age, gender, race, marital status, social class
  - Illness course - duration of illness, hospitalizations, prior treatments, treatment response
  - Personal factors - expectations of treatment, attitudes toward medication/other treatments, prior adherence

Refusal Reasons

- Example options
  - Experimental research study
  - randomized treatment
  - denial of need for treatment
  - denial of illness
  - study length
  - desire for treatment in another setting
  - uncooperativeness

Mechanics of Randomization: I

- Threats to Randomization
  - Assignment is known before individual is randomized
  - Assignment is not well concealed - the envelope please
  - In a non-blind study, participant withdraws once assignment is known
  - Randomization code can be broken by an interested party

Mechanics of Randomization: II

- Avoiding the threats:
  - Always
    - **Randomization lists prepared in advance**
    - **Record subject number on randomization form**
  - Multi-center studies
    - Centralized randomization Unit
  - Single Site Studies
    - **The envelope please**

“Housekeeping” Forms: I

- Visit form checklist
  - study time point
  - date
  - completed
    - yes
    - no, why not
  - procedures or other forms completed

“Housekeeping” Forms: II

- Medication Log - Strategies
  - Record medication changes
    - Start, dose change, discontinuation
  - Record dosage at fixed time points
    - Match assessment points

“Termination” Form

- Date of Last Visit
- Date of Study Termination
- Reason for Termination
  - Completed study
  - Worsening of clinical condition
  - Withdrawal of consent
  - Medical reason unrelated to target condition
  - Became ineligible
  - Other

Quality of Life Assessment

- General instruments to provide comparability to other RCT's
  - SF 36
- Specific instruments to address quality of life issues for the population/question
  - physical mobility in spinal cord injury patients
  - school setting in children

Adverse Events

- Every RCT should record adverse events
- General recording instruments readily available
- Specific events that may be related to population may also be indicated

Summary

- Design and choice of assessment instruments defines and RCT
- The assessment strategy brings the hypotheses and design to life
- The details of operation insure the integrity of a trial
- Multi-center trials magnify these needs